EXHIBIT 2

Professor Steve Wilton

PhD, BSc (Hons)

Foundation Chair in Molecular Therapy

About me

Our group is collaborating with Sarepta Therapeutics and the suite of antisense oligomers targeting the dystrophin gene have been licensed through UWA to Sarepta Therapeutics, Cambridge, MA. I am currently extending the therapeutic applications of antisense oligomers to other inherited and acquired conditions including spinal muscular atrophy, cystic fibrosis, multiple sclerosis, Alzheimer's, Pompe's disease, congenital muscular dystrophy, Huntington's and asthma.

I joined the Australian Neuro-muscular Research Institute (now Western Australian Neurosciences Research Institute-WANRI-Perth, Western Australia) in 1991. The concept of dystrophin exon skipping to treat Duchenne muscular dystrophy evolved during my development of diagnostic screening for neuromuscular diseases. Our group has been at the forefront in developing exon skipping as a therapy for Duchenne muscular dystrophy. We were the first to report specific dystrophin exon skipping in the *mdx* mouse (1999) and published the only panel of splice switching oligomers for every dystrophin exon (2007). We identified delivery as the *in vitro* limitation of the phosphorodiamidate morpholino oligomers as splice switching agents (2003). Other preclinical animal work included the phenotypic rescue of the severely dystrophic *dko* (dys^{-}/utr^{-}) mouse (2009) and specific induction of double exon skipping in the GRMD model of muscular dystrophy (2006). Our research has shown the morpholino oligomer chemistry was the more potent compound for *in vivo* exon skipping.

Our group designed the sequence of the morpholino oligomer (Eteplirsen), now in extended Phase 2b clinical trials in the USA. Our work has been recognized by the '2012 Western Australian Innovator of the Year' award, and more recently the '2013 Australian Museum Eureka Award for Translational Medicine'. I was appointed Director of WANRI in February 2013, and took up the Foundation Chair in Molecular Therapies at Murdoch University in March 2013 and am the head of Molecular Genetic Laboratory in the Centre for Comparative Genomics, Murdoch University.

Teaching area

Research areas

- Molecular genetics
- Antisense oligonucleotide technologies
- Gene Therapy
- Genetic Therapies
- Exon skipping
- Splice switching
- Muscle repair and regeneration
- Inherited neuromuscular diseases
- Duchenne muscular dystrophy
- Spinal muscular atrophy

Current projects

2014 - 2016

NHMRC Grant ID: 1062740

Investigators: Hool L, Fletcher S, Wilton SD

Project Title: The L-type calcium as a reporter of successful morpholino oligomer therapy in treatment of Duchenne Muscular Dystrophy cardiomyopathy

2013 - 2015

Sarepta Therapeutics Contract Research

Investigators: Wilton SD, Fletcher S

Contact me

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Fellow researchers

Useful links

Latest news

Research School

Project Title: Developing a splice switching therapy to correct a common defect in GAA causing adult onset Glycogen storage disease II

2013 - 2015

Sarepta Therapeutics Contract Research

Investigators: Wilton SD, Fletcher S

Project Title: Correlation study: PMO relative activity ranking in DMD patient myoblasts and normal myoblasts

2013 - 2017

NHMRC European Union

Project Title: RD-CONNECT: An integrated platform connecting registries, biobanks and clinical bioinformatics for rare disease research

2013 - 2016

MDA USA

Investigators: Wilton SD, Fletcher S, Bellgard MI

Project Title: Oligomer design & validation for DMD: quantum improvements in exon skipping

2013

Multiple Sclerosis Research Australia

Investigators: Wilton SD, Fletcher S

Project Title: Antisense oligomer induced suppression of target genes implicated in Multiple Sclerosis

2013 - 2015

NHMRC Grant ID: 1043758

Investigators: Bellgard MI, Wilton SD, Fletcher S

Project Title: Optimization of splice switching therapies to treat Duchenne muscular dystrophy

Awards and grants

AWARDS

2013
Awarded Eureka Prize for Medical Research Translation
2012
Awarded Western Australian Innovator of the Year 2012 (Fletcher, Wilton, UWA)

CURRENT GRANTS

2014 - 2016

NHMRC Grant ID: 1062740

Investigators: Hool L, Fletcher S, Wilton SD

Project Title: The L-type calcium as a reporter of successful morpholino oligomer therapy in treatment of Duchenne Muscular Dystrophy cardiomyopathy

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2013 - 2015

Sarepta Therapeutics Contract Research

Investigators: Wilton SD, Fletcher S

Project Title: Correlation study: PMO relative activity ranking in DMD patient myoblasts and normal myoblasts

2013 - 2017

NHMRC European Union

Investigators: Nolan D, Hammond E, Wilon SD, Fletcher S, Mallal S, Bellgard MI, Dawkins H, Goldblatt J, Baynam G, Weeramanthri T

Project Title: RD-CONNECT: An integrated platform connecting registries, biobanks and clinical bioinformatics for rare disease research

2013 - 2016

MDA USA

Investigators: Wilton SD, Fletcher S, Bellgard MI

Project Title: Oligomer design & validation for DMD: quantum improvements in exon skipping

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Multiple Sclerosis Research Australia

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PREVIOUS GRANTS

2012

MDWA -Team Spencer

Investigators: Wilton SD, Fletcher S

PhD Scholarship Loren Price

2011

UWA Research Development Awards 2011

Investigators: Adkin C, Wilton SD, Fletcher S

Project Title: Bypassing mutations in the S region of the Dystrophin gene by promoting alternative translation initiation codons using antisense oligonucleotides

2010-2012

Duchenne Ireland

Investigators: Wilton SD, Fletcher S, Ohlendieck, Adkin C

Project Title: Personalized Exon skipping Strategies for the treatment of DMD

2010-2012

NHMRC Grant ID: 634485

Investigators: Wilton SD, Fletcher S, Pinniger G

Project Title: Definition of dystrophin functional domains according to exon boundaries to optimize splice switching therapies for DMD

2010 - 2013

MDA USA

Investigators: Wilton SD

Project Title: A Splice switching strategies to treat Duchenne muscular dystrophy

2010 - 2012

SMA Australia

Investigators: Wilton SD, Fletcher S

2010-2011

SMA Europe - AFM

Investigators: Wilton SD, Fletcher S

Project Title: Antisense oligomer induced restoration of SMN expression as a therapy for Spinal Muscular Atrophy

2010

AFM

Investigators: Wilton SD, Fletcher S

Project Title: Suppression of DUX4 protein expression by antisense strategies

2010

Sir Charles Gardiner Hospital

Investigators: Wilton SD, Fletcher S

Project Title: Cell Banking for Western Australian Patients with DMD

2009 - 2013

NIH Grant ID: 2R01 NSO44146-05A1

Investigators: Wilton SD, Fletcher S

Project Title: Antisense oligonucleotide suppression of non-deletion DMD causing mutations

2009-2011

Gavriel Meir Trust

Investigators: Wilton SD, Fletcher S

Project Title: Exon Skipping project focusing on out-of-frame duplications

2009

Suneel's Light

Investigators: Wilton SD, Fletcher S

Project Title: Developing Exon Skipping strategies for Duchenne muscular dystrophy

2008-2010

The James & Matthew Grant Foundation

Investigators: Wilton SD, Fletcher S

Project Title: Refinement of exon skipping strategies to address mutations occurring in functional domains

2007-2010

MDA USA Grant ID: MDA4352

Investigators: Wilton SD, Fletcher S

Project Title: Refined AO design for enhanced dystrophin exon skipping.

2007-2009

Charley's Fund Inc.

Investigators: Wilton SD, Fletcher S

Project Title: The treatment or prevention of DMD or any other muscular dystrophy.

2007-2009

Muscular Dystrophy Ireland

Investigators: Wilton SD, Fletcher S

Project Title: Refinement of exon skipping strategies to address mutations occurring in functional domains of dystrophin

2006-2008

Parent Project United Kingdom

Investigators: Wilton SD, Fletcher S

Project Title: Demonstration of Antisense Oligonucleotide induced exon skipping in human muscle.

2004-2006

NHMRC Grant ID: 303216

Investigators: Wilton SD, Fletcher S

Project Title: Therapeutic induction of dystrophin positive revertant fibres in the mdx mouse

2004-2006

MDA USA Grant ID: MDA3718

Investigators: Wilton SD, Fletcher S

Project Title: Reducing the severity of DMD by redirecting pre-mRNA splicing.

2003-2007

NIH Grant ID: 1 R01 NS044146-02

Investigators: Wilton SD, Fletcher S

Project Title: Antisense oligonucleotide suppression of DMD

2002-2004

action benni & co e.v. PP (Germany)

Investigators: Wilton SD, Fletcher S

Project Title: Antisense oligonucleotide modifications to enhance specific exon skipping.

2002-2003

Parent Project, MDA USA

Investigators: Wilton SD

Project Title: Dusty Brandom Post-Doctoral Fellowship: to establish young investigators in DMD Research

2001-2003

NHMRC Grant ID: 139041

Investigators: Wilton SD, Fletcher S

Project Title: Minimizing consequences of DMD mutations using antisense oligonucleotides.

2001-2003

MDA USA Grant ID: MDA3099

Investigators: Wilton SD, Fletcher S

Project Title: Antisense oligonucleotide based genetic therapy for DMD

2001

MEDWA

Investigators: Wilton SD, Fletcher S

Project Title: Minimising the consequences of point mutations in the dystrophin gene

2000

MEDWA

Investigators: Wilton SD, Fletcher S

Project Title: Overcoming point mutations in the dystrophin gene

1998-2000

MDA USA Grant ID: MDA2527

Investigators: Wilton SD, Fletcher S

Project Title: Suppressing the gene defects in Muscular Dystrophy

1997-1999

NHMRC Grant ID: 970134

Investigators: Wilton SD, Fletcher S, Howell JMC, Kakulas BA

Project Title: Experimental gene therapy for the treatment of a model of DMD

Events and speaking engagements

2014

- Griffiths University "Gene therapy seminar: A clinical trial updated on Exon skipping and Duchenne muscular dystrophy" 4 Feb 2014 Brisbane, Australia
- Using FDASIA to Save Children with Duchenne Muscular Dystrophy, (Duchenne muscular dystrophy and exon skipping 101" 7 February 2014
- MDWA The Duke of Edinburgh's International Award 9 "Research & Science the impact of the condition" April 2014 Perth, Australia
- 5th FIP Pharmaceutical Sciences World Congress "Personalised genetic medicines for inherited disorders" 13-16 April 2014, Melbourne, Australia.
- 16th Annual TIDES Oligonucleotide and Peptide Therapeutics From Research through Commercialisation "Targeted Therapeutic Alternative Splicing" 12-15th May 2014, Rhode Island, USA

2013

- 34th Annual Lorne Genome Conference "Time makes more sense in Duchenne muscular dystrophy: Extended exon skipping treatment shows clinical benefits in Phase 2b studies" 17-19 February 2013, Victoria, Australia
- International Society for Cellular Therapy "Plenary Speaker" 22-25 April 2013 Auckland, New Zealand
- Parent Project Muscular Dystrophy Connect Conference "Panelist" 27-30 June 2013, Baltimore, USA
- 18th International World Muscle Society, 1-5 October 2013, California, USA
- The 4h Australia-China Biomedical Research Conference "An update on DMD exon skipping trials: making more sense with splice switching antisense oligonucleotides" 10-13 October 2013, Hangzhou, China

- Griffith University "Therapeutic alternative splicing: starting to make more sense in Duchenne muscular dystrophy". 28 November 2013, Gold Coast, Australia

2012

- Indian Ocean Rim Muscle Colloquium. "Duchenne muscular dystrophy and exon skipping: An update on clinical trials". 6-8 February 2012, Bangalore, India
- John Curtin School of Medical Research. "Restoration of dystrophin synthesis in Duchenne Muscular Dystrophy: from a "party trick" to Phase 2 clinical trials in less than a decade". 13 April 2012, Canberra, Australia
- The University of Hong Kong. "Splice switching therapies for Neuromuscular Diseases" 4 June 2012, Hong Kong
- Asian Oceanian Myology Center (AOMC). An overview of antisense oligomer induced splice switching to treat Duchenne muscular dystrophy and spinal muscular atrophy. 6-9 June 2012, Kyoto, Japan
- University of Technology Sydney Invited Seminar. "Splice intervention therapies for inherited disorders: from Thalassemia to Duchenne muscular dystrophy: Spinal muscular atrophy and ??". 23 August 2012, Sydney, Australia
- The University of Adelaide, "Splice intervention therapies to treat inherited disorders". 31 August 2012, Adelaide, South Australia
- Riding the wave. "Spinal Muscular Atrophy: Research into splice switching therapies DMD & exon skipping: Eteplirsen trial update". 6 October 2012, Brisbane, Australia
- World Muscle Society. Industry Symposium (Chair and speaker) "History of Exon Skipping and DMD" 9-13 October 2012, Perth, Australia
- Murdoch University. Institute for Immunology & Infectious Diseases Seminar. "Personalized medicine is becoming a reality for Duchenne muscular dystrophy". 22 November 2012, Perth, Australia
- OMICS2012. "Splice intervention therapies for Duchenne muscular dystrophy: from a concept to Phase 2b clinical trials". 26-28 November 2012, Fremantle, Australia
- Action Duchenne Meeting. "Restoring the dystroglycan complex with antisense oligomers: an international perspective. & Takin' charge workshop: Research, Care and management". 9-10 November 2012, London, UK
- University of Mons. Invited Seminar "Splice intervention therapies to treat inherited disorders". 3 December 2012 Mons, Belgium
- 194th ENMC workshop on Exon skipping. Invitation only. "Multiple exon skipping". 7-9 December 2012, Naarden, Netherlands

2011

- Lorne Genome Conference. Session Chair. 13-15 February 2011, Victoria, Australia
- Duchenne Parent Project, "Updates of Exon Skipping". 19 February 2011, Rome, Italy
- MDA's National Scientific Meeting, Keynote Presentation, "Splice switching therapies for Neuromuscular Diseases". 13-16 March 2011, Las Vegas, USA
- UK Neuromuscular Translational Research Conference 2011, Poster, "Transient mouse models for the preclinical evaluation of therapeutic dystrophin exon skipping strategies". 29-30 March 2011, London, UK
- Australasian Gene Therapy Society 7th Meeting, "Personalized Genetic Therapies for Neuromuscular Diseases, 4-6 May 2011, Melbourne, Australia
- Roland Forum for Brain Research 2011, "Splice switching therapies to treat genetic disorders: Trials and tribulations". 17 May 2011, Hebrew University Jerusalem
- Corner Stone Class, Mount Scopus Campus, "Personalized genetic medicine: can it become a reality? Invited Lecture,19 May 2011, Hebrew University Jerusalem
- Science in the Cinema 2011, Expert Panelist, 9 June 2011, West Perth, Australia
- PathWest Core Clinical Pathology & Biochemistry, Royal Perth Hospital, 9 August 2011, Perth, Western Australia
- 2nd Joint Symposium on Translational Medicine. "Personalized molecular surgery using splice switching antisense oligomers". 23-26 October 2011, Shanghai, China
- 9th International Annual Duchenne Conference, "Introduction to Exon Skipping". 4-5 November 2011, London, UK
- Treat-NMD Conference 2011, "Chaired - Antisense Technologies - Strategies and Successes". 8-11 November 2011, Geneva, Switzerland
- ActionDuchenne Time to stop wasting. "Exon skipping - improving delivery and efficiency". 12-13 November
- Institute for Immunology & Infectious Diseases Seminar, "Personalized medicine is becoming a reality for Duchenne muscular dystrophy". 22 November 2011, Murdoch University, Perth, Australia
- UWA Faculty of Medicine, Dentistry & Health Sciences Research Day, "Centres of Research Excellence - Proposals II". 24 November 2011, Perth, Australia
- RNA & Oligonucleotide Therapeutics, "Exon skipping and Duchenne muscular dystrophy - Pushing the boundaries". 4-7 December 2011, Gold Spring Harbor, New York, USA
- Center for Gene Therapy, The Research Institute, Nationwide Children's Hospital, Invited, "Molecular Scalpels for Splice Switching Compensation of Selected Genetic Disorders: Do we really know what is happening?". 8-11 December 2011, Columbus, USA

2010

- Symposium on Alternative Splicing in Neurodegenerative Diseases and Caner, "Antisense therapies for Duchenne MD: From revertant fibres to Becker MD". 7-9 February 2010, Tel Aviv, Israel
- Hebrew University Medical Centre, "What Can We Expect From Exon Skipping to Treat DMD Mutations?". 11 February 2010, Hadassah, Jerusalem
- Parent Project Muscular Dystrophy "A Panorama of Gene and Genetic Therapies for DMD". 13 February 2010, Rome, Italy
- Neuromuscular Disorders Conference - Towards a Brighter Future, "An Exon Skipping Update: Prosensa and MDEX Clinical Trials" & "Splice therapies: Genetic bandaids for SMA". 26-27 February 2010, Sydney, Australia

- World Congress of Internal Medicine, "Molecular By-pass Surgery: rescuing expression from defective genes". 20-25 March 2010, Melbourne, Australia
- The 4th Margaret River Region Forum: Pathways toward Molecular & Cellular Therapy, "Molecular Therapy for DMD". 28 April - 1 May 2010, Margaret River, Western Australia
- Parent Project Muscular Dystrophy: Leading the Fight to end Duchenne. 24-27 June 2010, Denver, USA
- DMD Therapeutic Development Meeting, "Skipping Rare Exons". 25-26 June 2010, Denver, USA
- World Federation of Neurology XII International Congress on Neuromuscular Diseases, "Clinical development of morpholinos for exon skipping in DMD". 17-22 July 2010, Naples, Italy
- MD2010 Connect Learn Share, "Genes and Disease". 9-10 September 2010, Perth Western Australia
- Huntington's Science Day, "Manipulating Gene Expression: Definitely for Duchenne MD, Maybe for Huntington's Disease?". 16 September 2010, Perth, Western Australia
- G'Day UK Life Sciences Event, "Personalised Genetic Therapies for Neuromuscular Disorders". 22 September 2010, London, UK
- Westmeade Children's Medical Research Institute, "Personalised splice switching therapies for Neuromuscular Therapies". 7 October 2010, Sydney, Australia
- Action Duchenne: It's time to Stop Wasting!, "Exon skipping - improving delivery and efficiency". 12-13 November 2010, Canary Wharf, London
- The Victor Chang Cardiac Research Institute 12th International Symposium: Charting the depths of RNA: from molecules to therapies, "Redirecting splicing for neuromuscular diseases: Clinical trials and future directions". 19 November 2010, Sydney, Australia
- Australian Health & Medical Research Council. "Targeted Splices switching intervention for DMD, Trials Triumphs and Tribulations". 17 November 2010, Melbourne, Australia

2009

- Department of Pediatrics, Kobe University Graduate School of Medicine. "Special Symposium on Exon Skipping." 9 November 2009, Kobe, Japan
- Combined Meeting of Antisense Society of Japan. "What can be expected from skipping dystrophin mutations?." 4-6 November 2009, Fukuoka, Japan
- Muscular Dystrophy Association Ireland. "Gene Therapy Seminar." 19 October 2009, MUI Maynooth, Ireland
- Action Duchenne Annual Conference. "Chain Workshop on Exon Skipping." 23-24 October 2009, London, United Kingdom
- Clinical Translational Research Seminar. "Trials and tribulations of splice switching Morpholinos for Duchenne Muscular Dystrophy." 16 September 2009, Melbourne, Australia
- 14th International Congress of the World Muscle Society. "Dystrophin Exon Skipping: What can we expect?". 9-12 September 2009, Geneva, Switzerland
- 19th Combined Biological Sciences Meeting. 28 August 2009, Perth, Western Australia
- Parent Project Muscular Dystrophy. "Meet the Experts" (Skype Presentation). 26-28 June 2009, Alanta, USA
- Cell Organization and Morphogenesis Symposium, 15 & 16 June 2009, Biopolis, Singapore
- 8th Asian & Oceanian Myology Centre (AOMC) Scientific Meeting. "Exon skipping and Duchenne muscular dystrophy: Hope, hype and how feasible?" 23-24 May 2009, Mumbai, India
- Muscular Dystrophy Association of New Zealand 2009 Window on Tomorrow, "Update in Molecular Therapies". 7-9 May 2009, Auckland, New Zealand
- HGSA "Splice Intervention to treat Duchenne Muscular Dystrophy and Beyond." 2-6 May 2009, Fremantle, Western Australia
- Australasian Gene Therapy Society 6th Meeting, "Splice switching therapies as personalized genetic treatments: applications to thalassemia, muscular dystrophy and spinal muscular atrophy." 29 April - 1 May 2009, Sydney, Australia
- LIWA 2009 Lung and Biological Science Symposium, "Splice manipulation as a therapy for Duchenne muscular dystrophy." 12-13 March 2009, Perth, Australia
- Lumen Christi College. "Molecular Genetic Therapy and Muscular Dystrophy." 19 February 2009, Perth, Australia
- Bone and Mineral Research Group Journal, "Splice manipulation therapies for DMD: opportunities and challenges." 17 February 2009, Perth, Australia
- Indian Ocean Rim Muscle Colloquium 2009. "Challenges facing exon skipping trials for Duchenne muscular dystrophy." 21-23 January 2009, Perth, Australia

2008

- AOMC, 7th Annual Scientific Meeting of the Asian Oceanian Myology Center, "Genetic treatments for the muscular Dystrophies." 13-14 November, Melbourne, Australia
- Information MD 2008, "Genetic Bandaids to reduce the severity of DMD." 13-14 November 2008, Melbourne, Australia
- Muscular Dystrophy Foundation Nepal 3rd Seminar in International Level on Duchenne Muscular Dystrophy Disables. "Genetic and small molecule therapies for muscular dystrophy." 8-9 November 2008, Nepal
- Action Duchenne 6th Annual Duchenne Conference, "Exon Skipping." 31 October - 2 November 2008, London, UK
- Oligonucleotide-Directed Splicing: Therapeutic Strategies, " Oligo design and elvaluation." 14-17 October 2008, The Banbury Center, Cold Spring Harbor Laboratory, New York, USA
- Suneel's Light "Developing Exon Skipping strategies for Duchenne muscular dystrophy." October 2008, Buffalo, USA
- Genzyme, "Invited splice manipulation therapies opportunities and challenges." October 2008, Boston, USA
- World Muscle Society 13th International WMS Congress. "Splice manipulation therapies: opportunities and challenges." 29 September- 2 October 2008, Newcastle, UK

- Therapeutic Goods Administration Seminar. " Exon Skipping to treat Duchenne Muscular Dystrophy a personalized genetic therapy." 21 August 2008, Canberra, Australia
- Human Genetics Society of Australasia 32nd Annual Scientific Meeting. 2-6 August 2008, Glenelg, South Australia
- Parent Project Muscular Dystrophy 2008 Annual Conference, "Overview of Research Strategies." 17-20 July 2008, Philadelphia, USA
- Bio International Convention, "New Therapeutic Modalities: Opportunities and Challenges." 17-20 June 2008, San Diego, USA
- The 3rd International Congress of Myology 2008, "Splice intervention therapies for muscle diseases" 26-30 May 2008, Marseille, France
- Parent Project Muscular Dystrophy, " Exon skipping for everyone: Where are we now and what to expect." 16 February 2008, Milan, Italy

2007

- The Third Australian Biotherapeutic and Regenerative Medicine Forum. "Antisense oligomers: personalized genetic therapies for Duchenne muscular dystrophy." 29 November - 1 December 2007, Margaret River, Western Australia
- 12th World Congress on Advances in Oncology and 10th International Symposium on Molecular Medicine. "Molecular by-pass surgery: Genetic intervention for Duchenne muscular dystrophy." 11-13 October 2007, Crete, Greece
- Neuro 2007. "How do genes work? How can we fix the bad ones?" 24 August 2007, Perth Western Australia
- Northwestern University Fienberg School of Medicine, Department of Neurology, "An antisense Renaissance: Exon Skipping and the Dystrophiin Gene" 16 July 2007, Chicago, USA
- Parent Project Muscular Dystrophy. "Overview of Research Strategies" 13-15 July 2007, Philadelphia, USA
- American Society of Gene Therapy. "Antisense oligonucleotide induced exon skipping as a therapy for Duchenne muscular dystrophy" 30 May - 3 June 2007 Seattle, Washington, USA
- WAIMR Seminar, "An Antisense Renaissance: redirecting gene expression patterns" 20th June 2007, Perth, Australia
- American Society of Gene Therapy "Antisense oligonucleotide induced exon skipping as a therapy for Duchenne muscular dystrophy." 30 May - 3 June 2007 Seattle, Washington, USA
- Centre for Neuromuscular and Neurological Disorders. "Clinical genetics of epilepsy and genetic approaches to treatment" 25 May 2007, Perth, Australia
- FDA/DIA Industry and Health Authority Conference on: Oligonucleotide-based Therapeutics. "Using antisense agents to alter splicing in Duchenne's Muscular Dystrophy: Can antisense provide individualized therapy?" 19-20 April 2007, Bethesda, USA
- MD2007, "Genetics in a nut shell: a little of what we know. & Exon skipping as a therapy of DMD" 13-14 April 2007, Perth. Australia
- 13th Australian Society of Cytogenetics. "An RNA Renaissance" 16-18 March 2007 Sydney, Australia
- 1st Molecular Genetics Society of Australasia Meeting, "RNA splicing and Murphy's Law: If something can go wrong it will." 16-18 March 2007, Sydney, Australia
- 149th ENMC International Workshop: planning phase I/II Clinical Trials using systemically delivered Antisense Oligonucleotides in Duchenne Muscular Dystrophy. 23-25 February 2007, Naarden, The Netherlands

2006

- Monaco Round Table Workshop. "Preclinical studies: Antisense oligonucleotide design" 14-15 January, 2006, Monaco
- Lorne Genome Conference. "Making sense from nonsense: manipulating splicing patterns with antisense oligonucleotides". 12-15 February, 2006. Lorne, Australia
- Special Seminar CRI. "Exon skipping for DMD: Hopes, Hype and the Future. 30 March, 2006 Children's Research Institute, Columbus Ohio USA
- A Mikellides Memorial Lecture "Gene medicines for muscular dystrophy: a light at the end of the tunnel?" 6 April, 2006. CING Nicosia, Cyprus
- Cyprus MDA Conference. "Exon skipping and the dystrophin gene transcript". 8 April, 2006 Cyprus Institute of Neurology and Genetics, Nicosia, Cyprus
- 3rd Singapore International Neurosciences Conference. " Addressing Neuromuscular Disorders With Antisense Oligonucleotides: Where Are The Limits?" 23-24 May,2006. Singapore
- XI International Congress of Neuromuscular Diseases. "Exon Skipping: A Genetic Therapy for DMD." 2-7 July 2006. Istanbul, Turkey
- Parent Project Muscular Dystrophy Annual Conference. "Duchenne 101" 14-16 July, 2006. Cincinnati
- Genetic Research Academic Industry Partnership Conference. "Antisense Induced Exon Skipping" 3-4 August 2006, Perth, Australia
- 11th International Congress of Human Genetics. "Making Sense in Spite of Nonsense: a splice Intervention Therapy for Duchenne Muscular Dystrophy" 6-10 August, 2006, Brisbane, Australia
- Combio 2006. "Molecular by-pass surgery: antisense oligonucleotides and the dystrophine gene" 24-28 September 2006, Brisbane, Australia
- United Kingdom Parent Project. "Exon skipping and Duchenne Muscular Dystrophy: A light at the end of the tunnel" 20-22 October, 2006. London
- Parent Project Australia Conference. "Exon skipping and DMD: Where are we now?" 28-29 October, 2006. Brisbane
- Unilever, "What is DMD, what is being done to treat it, what are the opportunities that funding provides" 2 November 2006, Sydney, Australia
- American Epilepsy Society Conference. "Splice Intervention Therapies." 1-5 December 2006, San Diego

2005

- PPMD sponsored AON Meeting. "Antisense oligo targets and chemistries for clinical trials." 30-31 January. 2005. Washington USA
- MDA USA TRAC meeting. "Update on Antisense oligonucleotide studies". 4th February, 2005. Tucson, USA
- Genetic Pathology Club, King Edward Memorial Hospital. "The brave new world of splice enhancer and suppressor elements". 9th March, 2005. Perth, Australia
- TIGEM Seminar Series. "Exon skipping: more than just making sense with DMD." 4th May, 2005. Naples, Italy
- Catholic University Seminar Series. "Exon skipping: more than just making sense with DMD." 5th May, 2005. Rome, Italy
- International Scientific Congress Myology 2005. "Exon skipping and DMD: A light at the end of the tunnel?" 13th - 17th May, 2005. Nantes, France
- Parent Project Muscular Dystrophy Annual Conference. "Genetics in a nutshell, a little of what we know" and "DMD and genetic velcro." 8-10 July, 2005, Cincinnati , USA
- MD2005 "Genetics in a nutshell, a little of what we know" and "DMD and genetic velcro."15-16 July, 2005, Perth, Western Australia
- 10th World Muscle Society Exon skipping and the dystrophin gene: Molecular by-pass surgery 28 September-1 October, 2005. Iguassu Falls, Brazil
- The Genetics Institute Special Seminar Making sense from the ashes of a defective dystrophin gene 17 October, 2005 Newcastle upon Tyne, UK
- PPUK Annual Seminar. Exon skipping and the dystrophin gene: a light at the end of the tunnel? 22-23 October, 2005 Russell Square, London, UK
- World Congress of Neurology. Exon skipping and the dystrophin gene: Starting to make more sense. Nov 5-11. 2005 Sydney, Australia
- MDA Clinical Directors Meeting Exon skipping and the dystrophin gene: A light at the end of the tunnel. 17-19 November, 2005 Tucson Arizona
- GSK Genetic Research Special Seminar. Manipulation of gene transcript splicing: application to simple and complex gene defects. 21 November, 2005, Raleigh, USA
- 41st National Turkish Neurology Congress, Exon skipping: molecular by-pass surgery for DMD. 5-10 December 2005 Istanbul, Turkey

2004

- Third Annual Scientific Meeting of the Asian & Oceanian Myology Center. "Antisense oligonucleotide induced changes in processing of gene transcripts". 8-9 January, 2004 Singapore
- Rescuing mRNA in DMD Roundtable Workshop. "Which antisense chemistry to use?". 17-18th, 2004. Monaco
- Gene Therapy Minisymposium. "Antisense oligos and neuromuscular disease. 18th February, 2004. University Department of Medicine, Perth, Australia
- Seminar series Department of Medical Technology & Physics. Sir Charles Gairdner Hospital, "Both ends of molecular medicine: High through-put genotyping and therapeutic manipulation of gene expression"7th April, 2004, Perth, Australia
- Australian & New Zealand Child Neurology Society Annual Meeting. "New therapies for Muscular Dystrophies". 7-9 May, 2004. Karriview Lodge, Margaret River, Western Australia
- Institute of Molecular Biosciences Seminar Series. "AOs and the dystrophin gene: from a party trick to clinical trials." 21st May, 2004. Brisbane, Queensland
- Muscular Dystrophy Association of South Africa. "Exon skipping from a party trick to clinical trials." and "Gene medicine: identification and manipulation" 29th June, 2004
- National Health Laboratory Services, University of Witwitersrand, Johannesburg, South Africa
- Parent Project Muscular Dystrophy Annual Conference. "Genetics in a nutshell, a little of what we know" and "DMD and genetic velcro." 9-11 July, 2004, Cincinnati , USA
- Directions for Muscular Dystrophy Conference 2004. "Genetics in a nutshell" and "Getting serious about antisense and DMD." 16th & 17th July, 2004 University of Southern Queensland, Toowoomba, Queensland
- Genetics and Population Health. "Re-directing pre-mRNA splicing using antisense oligonucleotides." 8-10 August, 2004. Fremantle, Western Australia
- Human Genetics Society of Australia Annual Meeting GenesWest. "From a party trick to clinical trials: A therapy for DMD" 11-13 August, 2004. Fremantle, Western Australia
- Royal Brisbane Hospital Seminar Series . "Manipulation of gene transcript splicing". 15th September, 2004. Video seminar
- Dept of Veterinary Biology & Biomedical Science Seminar Series. "Complex diseases and simple genetic therapies: all under one roof." 16th September, 2004. Murdoch University, Perth, Australia
- UK Parent Project Muscular Dystrophy Conference Annual Conference. "Starting to make some sense from a defective dystrophin gene: Bypassing Duchenne Mutations with "genetic velcro." 24th September, 2004. London, United Kingdom
- Stanford Dept. of Neurology and Neurological Sciences Seminar Series. "AOs and DMD: from the bench to the badminton court." 1st October 2004. San Francsico, USA
- University of Western Australia Genetic Epidemiology Course. "Fundamentals of Genotyping." 14th October, 2004. Perth, Australia
- University of Western Australia Agricultural Science Lecture. "Careers in Science... a non-logical progression."19th October, 2004. Perth, Australia
- ENMC workshop on Antisense oligonucleotides and DMD. "Choice of target sequences and Chemistries" 22-24th October, 2004. Naarden, Netherlands
- New Zealand Muscular Dystrophy Association Annual Meeting. "Genetics in a nutshell" and "Therapies under investigation to treat neuromuscular disorders" 3rd to 5th November , 2004. Auckland, New Zealand

- Asthma and Allergy Research Institute Margaret River Meeting. "Alternative splicing and therapeutic strategies." 11-13 November, 2004. Margaret River, Western Australia
- Australian Health and Medical Research Congress. "Antisense starts making sense in DMD." 26th November 2004, Sydney, Australia

2003

- Australian Society of Biochemistry and Molecular Biology 13th Annual Mundaring Weir Meeting. "Increased plasticity of gene expression through alternative splicing". 24th April, 2003. Perth, Australia
- Western Australian Institute for Medical Research Seminar. "Increased plasticity of gene expression through alternative splicing". 21st May, 2003. Perth, Australia
- XIX International Congress of Genetics. "Redirecting pre-mRNA splicing using antisense oligonucleotides". 6-11 July, 2003, Melbourne, Australia
- 2003 Parent Project Muscular Dystrophy. "Genetics in a nutshell: what you need to know" and "Redirecting expression of a DMD gene to restore dystrophin synthesis using "biological velcro". 11-13 July, 2003. Cincinnati, USA
- PMT Seminar Series, Department of Pharmacology. 15 July, 2003. "Both ends of molecular medicine: high throughput genotyping and redirection of gene expression". Lineberger Cancer Center, University of North Carolina, Chapel Hill, USA
- Special Seminar, Department of Human Genetics. "Antisense oligos and the dystrophin gene: starting to make some sense". 28 August,2003 . University of Utah, Salt Lake City, USA
- Invited Seminar, Genethon. "Modifying plasticity of gene expression using antisense oligonucleotides". 10 September, 2003. Paris, France
- Special Seminar, Department of Pharmacology and Toxicology. "Antisense oligos and the dystrophin gene: starting to make some sense". 11 September, 2003. University of Lausanne, Lausanne, Switzerland
- 2003 Italian Parent Project Meeting. "Genetics in a nutshell: what you need to know". "Redirecting expression of a DMD gene to restore dystrophin synthesis using "biological velcro". 19-20 September, 2003. Bologna, Italy
- Medical Research Seminar Series. "Redirecting Gene Expression for Fun and Profit". Asthma and Allergy Research Institute, 3 November, 2003. Perth, Australia

2002

- Human Genetics Society of Australasia WA Branch Lecture. "Antisense therapy for Duchenne Muscular Dystrophy: Two wrongs making it right." 19th February, 2002. Perth, Australia
- GlaxoSmithKline GEMS Scientific review. "High-through put genotyping of Turkey-11". SD Wilton, PA Akkari and R Duff. 12-13 March, 2002. Philadelphia, USA
- Montrose Access Therapies for Duchenne Muscular Dystrophy. "Antisense oligos and the dystrophin gene". 12 April, 2002. Brisbane, Australia
- Parents Project Meeting. "Basics of Genetics and Gene Expression". "Reducing the severeity of DMD using antisense oligonucleotides". 27-29 June, 2002, Pittsburg, USA
- AusBiotec 2002 Conference "Antisense oligonucleotide therapies: starting to make some sense". 18-21 August, Melbourne Australia
- Biomedical Engineering Society of WA. "Gene Screening and Therapy". 23rd September, 2002. Perth, Australia
- Western Australian Bone and Cartilage Program. "Both Ends of Molecular Medicine- Gene Discovery to Drug Design". 18 October, 2002. Perth, Australia
- WA Human Genetic Research Forum. "Molecular Medicine-Gene Discovery and Therapy" 24th October, 2002. Perth Australia
- 4th HUGO Pacific Meeting and 5th Asia-Pacific Conference on Human Genetics. "The basics of gene expression and potential therapies", "Antisense oligonucleotides and the dystrophin gene: starting to make some sense" October 27-30, 2002. Pattaya, Thailand
- Neurological Expo 2002. "Neurogenetics- A new world of progress". 2nd December, 2002. Perth, Australia
- 6th Meeting of the International Mesothelioma Interest Group. "Altering gene expression using antisense oligonucleotides". 1-4 December, 2002, Perth Australia

2001

- Perspectives to studies on myogenesis and therapeutics of muscular diseases in the 21st century. "Antisense induced dystrophin expression in the mdx mouse". SD Wilton. 22 March, 2001. Kyoto, Japan
- Opening Ceremony for the Animal Research Facility, National Institute of Neuroscience, National Centre of Neurology and Psychiatry. "Restoration of dystrophin expression in the mdx mouse using antisense oligonucleotides in a gene knock-in approach". 23 March, 2001. Tokyo, Japan
- Parents Project Annual Conference. "Restoration of dystrophin expression in the mdx mouse using antisense oligonucleotides". SD Wilton, CJ Mann, T Ly, S Fletcher, F Lloyd, J Morgan and TA Partridge. 22-24 June, 2001. Pittsburg, USA
- Glaxo Wellcome Scientific Review. Genetics of Metabolic syndrome. "The high throughput genotyping facility in Perth". SD Wilton. 20, June 2001. Boston, USA
- 7th Annual Meeting of the Japanese Society of Gene Therapy. "The application of antisense oligonucleotides in a "gene knock-in" therapy for Duchenne muscular dystrophy. CJ Mann, K Honeyman, F Lloyd, S Fletcher and SD Wilton. 5-7 July, 2001. Tokyo, Japan
- Genetic Research In Clinical Disease. Asthma and Allergy Research Institute. "Antisense oligonucleotides and Duchenne muscular dystrophy". 14th November, 2001. Perth, Australia

2000

- NIH Workshop on therapeutic approaches for DMD. "Antisense oligonucleotides and exon skipping in the dystrophin gene". SD Wilton. 15-16 May, 2000. Washington, USA

Professional and community service

PROFESSIONAL MEMBERSHIPS

- President, Australasian Gene Therapy Society, 2009-2013
- Executive Board, World Muscle Society, 2010-2012
- Editorial board, Acta Myologica
- Chief Editor, Acta Myologica, Dec 2005
- Steering committee, iDESC (International dystrophin exon skipping consortium).
- Editor, Oligonucleotides
- Editor, Acta Myologica
- Editor, Molecular Therapy-Nucleic Acids
- Member, MDEX consortium
- Member, Australian Society for Biochemistry and Molecular Biology.
- Member, Genetics Society of Australia.

COMMITTEE INVOLVEMENT & CONFERENCE ORGANISATION

- 2012 MD2012 Conference, Perth. Member of the oganising committee for a muscular dystrophy conference linking healthcare professionals, researchers and the public
- 2012 RD Connect workshop, Perth. Member of the organizing committee
- 2010 MD2010 Conference, Perth. Member of the oganising committee for a muscular dystrophy conference linking healthcare professionals, researchers and the public
- 2007 MD2007 Conference, Perth. Member of the oganising committee for a muscular dystrophy conference linking healthcare professionals, researchers and the public
- 2005 MD2005 Conference, Perth. Member of the oganising committee for a muscular dystrophy conference linking healthcare professionals, researchers and the public
- 2003 MD2003 Conference, Perth. Member of the oganising committee for a muscular dystrophy conference linking healthcare professionals, researchers and the public

Doctoral and masters supervisions

PhD Students

2013 - Present	Ianthe Pitout, Murdoch University
2012 - Present	Loren Price, UWA
2010 - 2014	Lucy Barrett, UWA
2010 - 2013	Robyn Luo, UWA
2005 - 2012	Mitrpant Chalermchai, Edith Cowan University
2009-2012	Arada Rojana-udomsart , UWA
2006-2010	Rachel Duff, UWA
2004-2006	Graham McClorey, UWA
2000-2003	Stephen Errington, Edith Cowan University
2000-2003	Christopher Mann, UWA
1997-2001	Marie McCluskey, Edith Cowan University

BSc Honours Students

2013	Charles Toh, Anatomy Physiology & Human Biology, UWA (1st Class)
2012	Soma Amin, Genetics (Biochemistry) UWA, (2A)
2009	Lucy Barrett, Genetics, UWA (1st Class)
2008	Leah Stone, UWA (1st Class)
2007	Naoibh McLoughlin, Genetics, UWA
2006	Heidi Madden, Genetics, UWA (1st Class)
2006	Catherine Coleman, UWA (1st Class)
2005	Mathew Welch, Murdoch University (2A)
2003	Clint Johnson, Pathology, UWA, (1st Class)
2003	Sara Thean, Genetics, UWA (2A)

2002	David Martino, Anatomy and Human Biology, UWA, (2A)
2002	Bianca Gebski, Pathology, UWA (2A)
2001	Mark Cruickshank, Pathology, UWA (1st Class)
1999	Hayley Durling, Edith Cowan University (1st Class)
1994	Rolee Kumar, Murdoch University (2A)
1992	David Darragh, Pathology, UWA (2B)
1991	Ricky Lareau, Pathology, UWA (2A)

Publications

Book Chapters

- Adkin C, Fletcher S and **Wilton SD**, Optimizing splice-switching oligomer sequences using 2'-O-methyl phosphorothioate chemistry, pp. 169-188 in Methods Mol Biol.
- **S.D Wilton**, Molecular Medicine: Potential therapies for genetic diseases, pp. 275-285 in Frontiers in Human Genetics:Diseases and Technologies, edited by Poh San Land Yap E. World Scientific Publishing Co. Pte. Ltd.
- **SD Wilton**, Multimedia methods in molecular biology, edited by Partridge TA. Chapman and Hall.
- Gregg K, **Wilton SD**, Rogers GE and Molloy PL, Manipulation and expression of genes in eukaryotes, pp. 65-72.

Fully Refereed Reviews

- Barrett LW, Fletcher S and **Wilton SD** (2012) Regulation of eukaryotic gene expression by the untranslated gene regions and other non-coding elements. Cell Mol Life Sci 69: 3613-3634.
- **Wilton SD**, and Fletcher S (2011) RNA splicing manipulation: strategies to modify gene expression for a variety of therapeutic outcomes. Curr Gene Ther 11: 259-275.
- Laing NG, Davis MR, Bayley K, Fletcher S and **Wilton SD** (2011) Molecular diagnosis of duchenne muscular dystrophy: past, present and future in relation to implementing therapies. Clin Biochem Rev 32: 129-134.
- **Wilton SD**, and Fletcher S (2010) Splice modification to restore functional dystrophin synthesis in Duchenne muscular dystrophy. Curr Pharm Des 16: 988-1001.
- Mitrpant C, Fletcher S and **Wilton SD** (2009) Personalised genetic intervention for Duchenne muscular dystrophy: antisense oligomers and exon skipping. Curr Mol Pharmacol 2: 110-121.
- **Wilton SD**, and Fletcher S (2008) Exon skipping and Duchenne muscular dystrophy: Hope, hype and how feasible? Neurol India 56: 254-262.
- **Wilton SD**, and Fletcher S (2006) Modification of pre-mRNA processing: application to dystrophin expression. Curr Opin Mol Ther 8: 130-135.
- **Wilton SD**, and Fletcher S (2006) Redirecting splicing to address dystrophin mutations: molecular by-pass surgery. Prog Mol Subcell Biol 44: 161-197.
- **Wilton SD**, and Fletcher S (2005) RNA splicing manipulation: strategies to modify gene expression for a variety of therapeutic outcomes. Curr Gene Ther 5: 467-483.
- **Wilton SD**, and Fletcher S (2005) Antisense oligonucleotides, exon skipping and the dystrophin gene transcript. Acta Myol 24: 222-229.
- Mcclorey G, Fletcher S and **Wilton S** (2005) Splicing intervention for Duchenne muscular dystrophy. Curr Opin Pharmacol 5: 529-534.
- Fletcher S, **Wilton SD** and Howell JM (2000) Gene therapy and molecular approaches to the treatment of hereditary muscular disorders. Curr Opin Neurol 13: 553-560.

Fully Refereed Journals

- **Wilton SD**, Fletcher S and Flanigan KM (2014) Dystrophin as a therapeutic biomarker: Are we ignoring data from the past? Neuromuscul Disord 24: 463-466.
- Luo YB, Mastaglia FL and **Wilton SD** (2014) Normal and aberrant splicing of LMNA. J Med Genet 51: 215-223.
- Greer KL, Lochmuller H, Flanigan K, Fletcher S and **Wilton SD** (2014) Targeted exon skipping to correct exon duplications in the dystrophin gene. Mol Ther Nucleic Acids 3: e155.
- Zhou H, Janghra N, Mitrpant C, Dickinson RL, Anthony K, Price L, Eperon IC, **Wilton SD**, Morgan J and Muntoni F (2013) A novel morpholino oligomer targeting ISS-N1 improves rescue of severe spinal muscular atrophy transgenic mice.
- Tremblay JP, Xiao X, Aartsma-Rus A, Barbas C, Blau HM, Bogdanove AJ, Boycott K, Braun S, Breakefield XO, Bueren JA, Buschmann M, Byrne BJ, Calos M, Cathomen T, Chamberlain J, Chuah M, Cornetta K, Davies KE, Dickson JG, Duchateau P, Flotte TR, Gaudet D, Gersbach CA, Gilbert R, Glorioso J, Herzog RW, High KA, Huang W, Huard J, Joung JK, Liu D, Liu D, Lochmuller H, Lustig L, Martens J, Massie B, Mavilio F, Mendell JR, Nathwani A, Ponder K, Porteus M, Puymirat J, Samulski J, Takeda S, Thrasher A, Vandendriessche T, Wei Y, Wilson JM, **Wilton SD**, Wolfe JH and Gao G (2013) Translating the genomics revolution: the need for an international gene therapy consortium for monogenic diseases. Mol Ther 21: 266-268.
- Rojana-Udomsart A, Mitrpant C, James I, Witt C, Needham M, Day T, Kiers L, Corbett A, Martinez P, **Wilton SD** and Mastaglia FL (2013) Analysis of HLA-DRB3 alleles and supertypical genotypes in the MHC Class II region in sporadic inclusion body myositis. J Neuroimmunol 254: 174-177.
- Rojana-Udomsart A, Mitrpant C, Bundell C, Price L, Luo YB, Fabian V, **Wilton SD**, Hollingsworth P and Mastaglia FL (2013) Complement-mediated muscle cell lysis: a possible mechanism of myonecrosis in anti-SRP associated necrotizing myopathy (ASANM). J Neuroimmunol 264: 65-70.

- Qiu W, Pham K, James I, Nolan D, Castley A, Christiansen FT, Czarniak P, Luo Y, Wu J, Garlepp M, **Wilton S**, Carroll WM, Mastaglia FL and Kermode AG (2013) The influence of non-HLA gene polymorphisms and interactions on disease risk in a Western Australian multiple sclerosis cohort. J Neuroimmunol 261: 92-97.
- Plummer PN, Freeman R, Taft RJ, Vider J, Sax M, Umer BA, Gao D, Johns C, Mattick JS, **Wilton SD**, Ferro V, Mcmillan NA, Swarbrick A, Mittal V and Mellick AS (2013) MicroRNAs regulate tumor angiogenesis modulated by endothelial progenitor cells. Cancer Res 73: 341-352.
- Pigozzo SR, Da Re L, Romualdi C, Mazzara PG, Galletta E, Fletcher S, **Wilton SD** and Vitiello L (2013) Revertant fibers in the mdx murine model of Duchenne muscular dystrophy: an age- and muscle-related reappraisal. PLoS One 8: e72147.
- Moorwood C, Soni N, Patel G, **Wilton SD** and Khurana TS (2013) A cell-based high-throughput screening assay for posttranscriptional utrophin upregulation. J Biomol Screen 18: 400-406.
- Mitrpant C, Porensky P, Zhou H, Price L, Muntoni F, Fletcher S, **Wilton SD** and Burghes AH (2013) Improved antisense oligonucleotide design to suppress aberrant SMN2 gene transcript processing: towards a treatment for spinal muscular atrophy. PLoS One 8: e62114.
- Luo YB, Mitrpant C, Johnsen RD, Fabian VA, Fletcher S, Mastaglia FL and **Wilton SD** (2013) Investigation of age-related changes in LMNA splicing and expression of progerin in human skeletal muscles. Int J Clin Exp Pathol 6: 2778-2786.
- Luo YB, Mitrpant C, Johnsen R, Fabian V, Needham M, Fletcher S, **Wilton SD** and Mastaglia FL (2013) Investigation of splicing changes and post-translational processing of LMNA in sporadic inclusion body myositis. Int J Clin Exp Pathol 6: 1723-1733.
- Luo YB, Johnsen RD, Griffiths L, Needham M, Fabian VA, Fletcher S, **Wilton SD** and Mastaglia FL (2013) Primary over-expression of AbetaPP in muscle does not lead to the development of inclusion body myositis in a new lineage of the MCK-AbetaPP transgenic mouse. Int J Exp Pathol 94: 418-425.
- Porensky PN, Mitrpant C, Mcgovern VL, Bevan AK, Foust KD, Kaspar BK, **Wilton SD** and Burghes AH (2012) A single administration of morpholino antisense oligomer rescues spinal muscular atrophy in mouse. Hum Mol Genet 21: 1625-1638.
- Fletcher S, Adkin CF, Meloni P, Wong B, Muntoni F, Kole R, Fragall C, Greer K, Johnsen R and **Wilton SD** (2012) Targeted exon skipping to address "leaky" mutations in the dystrophin gene. Mol Ther Nucleic Acids 1: e48.
- Anderton RS, Price LL, Turner BJ, Meloni BP, Mitrpant C, Mastaglia FL, Goh C, **Wilton SD** and Boulos S (2012) Co-regulation of survival of motor neuron and Bcl-xL expression: implications for neuroprotection in spinal muscular atrophy. Neuroscience 220: 228-236.
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- Vanderplanck C, Ansseau E, Charron S, Stricwant N, Tassin A, Laoudj-Chenivesse D, **Wilton SD**, Coppee F and Belayew A (2011) The FSHD atrophic myotube phenotype is caused by DUX4 expression. PLoS One 6: e26820.
- Pichavant C, Aartsma-Rus A, Clemens PR, Davies KE, Dickson G, Takeda S, **Wilton SD**, Wolff JA, Wooddell CI, Xiao X and Tremblay JP (2011) Current status of pharmaceutical and genetic therapeutic approaches to treat DMD. Mol Ther 19: 830-840.
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